Non-technical Abstract

Retinoblastoma is the most common primary malignant tumor of children and usually occurs in children under the age of 3 years. Current standard treatment for nonmetastatic retinoblastoma is enucleation. Although this results in a high rate of survival, enucleation results in blindness and severe cosmetic facial deformity. Recently, attention has been turned to finding alternative therapies that will result in a high cure rate but will allow salvage of the affected eye. Occasionally a child presents with a small tumor that can be eradicated with cryotherapy or laser photocoagulation while still preserving the eye and useful vision. Unfortunately, most children present with tumors that are too large for these types of therapies. In an attempt to shrink a larger tumor to a size that can be managed by these local therapies, clinical investigators have begun trials using systemic chemotherapy instead of enucleation. Although preliminary studies have shown promise, chemotherapy has significant side effects including an increased rate of second malignancies. Because patients with retinoblastoma have a significant second malignancy potential, especially bone cancer, as a natural course of their disease, an alternative therapy without systemic toxicity would be desirable.

Mice that have retinoblastoma were successfully treated using a gene therapy protocol. The tumors in most of the animals were either completely or nearly completely eradicated with this therapy. The goal of this study is to arrive at a safe dose of the gene therapy vector in children with retinoblastoma. Future studies will address whether this therapy will be useful in shrinking the tumors in children so their affected eye can be saved using local control measures.